

CLAIMS

What is claimed is:

- 5 1. A method for delivering recombinant AAV virions to a subject, comprising
administering via convection-enhanced delivery (CED) said rAAV virions into the CNS of the
subject, wherein said rAAV virions comprise a nucleic acid sequence encoding a therapeutic
polypeptide.
- 10 2. The method of claim 1, wherein the administering is done with an osmotic pump.
- 15 3. The method of claim 1, wherein the administering is done with an infusion pump.
4. The method of claim 1, wherein the nucleic acid sequence encodes an aromatic-
amino-acid decarboxylase (AADC).
- 20 5. The method of claim 1 wherein the subject is a human.
6. The method of claim 1, wherein the rAAV virions are administered into the striatum.
- 25 7. A method for delivering recombinant AAV virions to a subject having a CNS
disorder, comprising administering via convection-enhanced delivery (CED) said virions into the
CNS of the subject, wherein said virions comprise a nucleic acid sequence encoding a therapeutic
polypeptide.
8. The method of claim 7 wherein the CNS disorder is Parkinson's disease, the rAAV
virions are administered into the striatum and wherein the nucleic acid sequence encodes AADC

Sub 1B' 9. A method for treating a neurodegenerative disease in a subject, said method comprising:

(a) providing a preparation comprising recombinant adeno-associated virus (rAAV) virions, wherein said virions comprise a nucleic acid sequence that is expressible in transduced cells to provide a therapeutic effect in the subject; and

(b) delivering the preparation to the CNS of the subject using convection-enhanced delivery (CED), wherein said virions transduce neural cells and the nucleic acid sequence is expressed to provide a therapeutic effect in the subject suitable for treating said neurodegenerative disease.

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10. The method of claim 9, wherein the neurodegenerative disease is Parkinson's disease.

15 11. The method of claim 9, wherein the nucleic acid sequence expressible in transduced cells encodes AADC or functional fragment thereof.

12. The method of any one of claims 9-11, further comprising administering to the subject at least one additional therapeutic compound.

20 13. The method of claim 12 wherein the at least one additional therapeutic compound is L-dopa.

14. The method of claim 13, further comprising administering L-dopa and, optionally, carbidopa to the subject.

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15. A method of determining levels of dopamine activity in the brain of subject comprising;

(a) administering a labeled tracer to the subject, wherein binding of the tracer to a cell is indicative of dopamine activity; and

(b) imaging the subject's brain to determine the number of cells which bind the labeled tracer, thereby determining levels of dopamine activity in the subject's brain.

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17. The method of claim 15, wherein the imaging is positron emission tomograph (PET) imaging.

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